Clinical trial results:
A Phase 3, Open-label, Non-controlled, Multi-dose Study to Evaluate the Pharmacokinetics, Safety and Tolerability, and Efficacy of Immune Globulin Subcutaneous (Human), 20% Solution (IGSC, 20%) in Japanese Subjects with Primary Immunodeficiency Diseases (PID)

Summary

EudraCT number	2022-001873-29
Trial protocol	Outside EU/EEA
Global end of trial date	20 December 2021

Results information

Result version number	v2 (current)
This version publication date	15 December 2022
First version publication date	06 July 2022
Version creation reason	

Trial information

Trial identification

Sponsor protocol code	TAK-664-3001

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04346108
WHO universal trial number (UTN)	U1111-1189-8055

Notes:

Sponsors

Sponsor organisation name	Shire
Sponsor organisation address	300 Shire Way, Lexington, United States, MA 02421
Public contact	Study Director, Shire, ClinicalTransparency@shire.com
Scientific contact	Study Director, Shire, ClinicalTransparency@shire.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	No
Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial?	No
Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial?	Yes

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	20 December 2021
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	20 December 2021
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The main objective of this study is to assess serum trough Immunoglobulin globulin G (IgG) concentrations following weekly administration of IGSC, 20% (Epoch 2) and serum trough IgG concentration after biweekly administration of IGSC, 20% in Japanese participants with PID.

Protection of trial subjects:

All the participants were required to read and sign the Informed Consent Form

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	11 August 2020
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Japan: 17
Worldwide total number of subjects	17
EEA total number of subjects	0

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	4
Adolescents (12-17 years)	2
Adults (18-64 years)	10
From 65 to 84 years	1
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

Participants took part in the study at 8 investigative sites in Japan from 11 August 2020 to 22 December 2021.

Pre-assignment

Screening details:

Participants diagnosed with primary immunodeficiency diseases (PID) received immunoglobulin administered intravenously (IGIV) in Epoch 1, followed by immunoglobulin administered subcutaneously (IGSC) 20% in Epoch 2 and then followed by Epoch 3 respectively for up to approximately 50 weeks.

Period 1

Period 1 title	Epoch 1 (13 weeks)
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Investigator, Monitor, Assessor, Subject

Arms

Arm title	Epoch 1: IGIV 200-600 mg/kg

Arm description:

Participants received 200 to 600 mg/kg of Immunoglobulin Intravenous (IGIV) infusion for every 3 or 4 weeks for up to 13 weeks.

Arm type	Experimental
Investigational medicinal product name	Immune Globulin Intravenous (IGIV)
Investigational medicinal product code	
Other name	Immune Globulin Infusion (Human)
Pharmaceutical forms	Infusion
Routes of administration	Intravenous use

Dosage and administration details:

Participants received IGIV infusion.

Number of subjects in period 1	Epoch 1: IGIV 200-600 mg/kg
Started	17
Completed	17

Period 2

Period 2 title	Epoch 2 (24 weeks after Epoch 1)
Is this the baseline period?	No
Allocation method	Randomised - controlled
Blinding used	Double blind

Roles blinded	Subject, Investigator, Monitor, Assessor

Arms

Arm title	Epoch 2: IGSC (20%) 50-200 mg/kg

Arm description:

Participants who entered to Epoch 2 from Epoch 1 received 50-200 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion once a week up to approximately 24 weeks after Epoch 1.

Arm type	Experimental
Investigational medicinal product name	Immune Globulin Subcutaneous, 20% Solution (IGSC, 20%)
Investigational medicinal product code	
Other name	Immune Globulin Infusion (Human)
Pharmaceutical forms	Infusion
Routes of administration	Subcutaneous use

Dosage and administration details:

Participants received IGSC, 20% SC infusion.

Number of subjects in period 2	Epoch 2: IGSC (20%) 50-200 mg/kg
Started	17
Completed	15
Not completed	2
Consent withdrawn by subject	1
Withdrawal by Parent/Guardian	1

Period 3

Period 3 title	Epoch 3 (12 weeks after Epoch 2)
Is this the baseline period?	No
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator, Monitor, Assessor

Arms

Arm title	Epoch 3: IGSC (20%) 100-400 mg/kg

Arm description:

Participants who entered to Epoch 3 from Epoch 2 received 100-400 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion biweekly up to approximately 12 weeks after Epoch 2.

Arm type	Experimental
Investigational medicinal product name	Immune Globulin Subcutaneous, 20% Solution (IGSC, 20%)
Investigational medicinal product code	
Other name	Immune Globulin Infusion (Human)
Pharmaceutical forms	Infusion
Routes of administration	Subcutaneous use

Dosage and administration details:

Participants received IGSC, 20% SC infusion.

Number of subjects in period 3[1]	Epoch 3: IGSC (20%) 100-400 mg/kg
Started	7
Completed	7

Notes:

[1] - The number of subjects starting the period is not consistent with the number completing the preceding period. It is expected the number of subjects starting the subsequent period will be the same as the number completing the preceding period.

Justification: Epoch 3 included those participants who completed Epoch 2 and were eligible to enter Epoch 3.

Subject analysis sets	
Subject analysis set title	Epoch 1: Immune Globulin Intravenous (IGIV)
Subject analysis set type	Full analysis
Subject analysis set description: Participants received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for up to 13 weeks.	
Subject analysis set title	Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC)
Subject analysis set type	Full analysis
Subject analysis set description: Participants who entered to Epoch 2 from Epoch 1 received 50-200 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion once a week up to approximately 24 weeks after Epoch 1.	
Subject analysis set title	Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Subject analysis set type	Full analysis
Subject analysis set description: Participants who entered to Epoch 3 from Epoch 2 received 100-400 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion biweekly up to approximately 12 weeks after Epoch 2.	
Subject analysis set title	Age 2-7 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Sub-group analysis
Subject analysis set description: Participants aged 2-7 years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.	
Subject analysis set title	Age 8-13years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Sub-group analysis
Subject analysis set description: Participants aged 8-13 years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.	
Subject analysis set title	Age 2-11years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Full analysis
Subject analysis set description: Participants aged 2-11 years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.	
Subject analysis set title	Age ≥12 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Full analysis
Subject analysis set description: Participants aged >=12 years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.	
Subject analysis set title	Age 2-12years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Full analysis
Subject analysis set description: Participants aged 2-12 years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a	

total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	Age ≥13 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Full analysis

Subject analysis set description:

Participants aged >=13 years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	Age 2-13years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Full analysis

Subject analysis set description:

Participants aged 2-13 years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	Age ≥14 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Full analysis

Subject analysis set description:

Participants aged 14 and above years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	IGSC: Epoch 2: 2-13 years
Subject analysis set type	Sub-group analysis

Subject analysis set description:

Participants aged 2-13 years received approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch2.

Subject analysis set title	IGSC: Epoch 2: >=14 years
Subject analysis set type	Sub-group analysis

Subject analysis set description:

Participants aged >=14 years received approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2.

Subject analysis set title	IGSC: Epoch 3: 2-13 years
Subject analysis set type	Sub-group analysis

Subject analysis set description:

Participants aged 2-13 years received approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	IGSC: Epoch 3: >=14 years
Subject analysis set type	Sub-group analysis

Subject analysis set description:

Participants aged >=14 years received approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	IGIV: Epoch 1: 3-week interval
Subject analysis set type	Full analysis

Subject analysis set description:

Participants received approximately 200 to 600 mg/kg of IGIV infusion for every 3 for a total of 13 weeks.

Subject analysis set title	IGIV: Epoch 1: 4-week interval
Subject analysis set type	Full analysis

Subject analysis set description:

Participants received approximately 200 to 600 mg/kg of IGIV infusion for every 4 for a total of 13 weeks.

Subject analysis set title	IGSC: Epoch 3: Biweekly

Subject analysis set type	Full analysis

Subject analysis set description:

Participants received approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 1.

Subject analysis set title	IGSC: Epoch 2
Subject analysis set type	Full analysis

Subject analysis set description:

Participants received approximately 50 to 200 mg/kg of IGSC infusion, 20% once a week for a total of 24 weeks after Epoch 1.

Subject analysis set title	IGIV 200 to 600 mg/kg + IGSC [50 to 200 and 100 to 400] mg/kg
Subject analysis set type	Full analysis

Subject analysis set description:

Participants received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks up to 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	Epoch 2: IGSC (20%) 50-200 mg/kg
Subject analysis set type	Full analysis

Subject analysis set description:

Participants who entered to Epoch 2 from Epoch 1 received 50-200 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion once a week up to approximately 24 weeks after Epoch 1.

Subject analysis set title	Epoch 3: IGSC (20%) 100-400 mg/kg
Subject analysis set type	Full analysis

Subject analysis set description:

Participants who entered to Epoch 3 from Epoch 2 received 100-400 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion biweekly up to approximately 12 weeks after Epoch 2.

Subject analysis set title	Epoch 1: IGIV 3-week Interval
Subject analysis set type	Full analysis

Subject analysis set description:

Participants received 200 to 600 mg/kg of Immunoglobulin Intravenous (IGIV) infusion for every 3 weeks for up to 13 weeks.

Subject analysis set title	Epoch 1: IGIV 4-week Interval
Subject analysis set type	Full analysis

Subject analysis set description:

Participants received 200 to 600 mg/kg of Immunoglobulin Intravenous (IGIV) infusion for every 4 weeks for up to 13 weeks.

Subject analysis set title	Epoch 2: IGSC, 20% Weekly
Subject analysis set type	Full analysis

Subject analysis set description:

Participants who entered to Epoch 2 from Epoch 1 received 50-200 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion once a week up to approximately 24 weeks after Epoch 1.

Subject analysis set title	Epoch 2: IGSC, 20% Weekly (EOS [Week 25]/ ET)
Subject analysis set type	Full analysis

Subject analysis set description:

Participants who entered to Epoch 2 from Epoch 1 received 50-200 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion once a week up to end of study (EOS) (24 weeks) or early termination (ET) after Epoch 1.

Subject analysis set title	Epoch 3: IGSC, 20% Biweekly
Subject analysis set type	Full analysis

Subject analysis set description:

Participants who entered to Epoch 3 from Epoch 2 received 100-400 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion biweekly up to EOS (13 weeks) after Epoch 2.

Subject analysis set title	Epoch 3: IGSC, 20% Biweekly (Week 13)
Subject analysis set type	Full analysis

Subject analysis set description:

Participants who entered to Epoch 3 from Epoch 2 received 100-400 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion biweekly up to EOS (13 weeks) after Epoch 2.

Reporting group values	Epoch 1: Immune Globulin Intravenous (IGIV)	Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC)	Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects	17	17	7
Age Categorical			
Units: Subjects			
Age continuous			
Units: years			
arithmetic mean			
standard deviation	±	±	±
Gender categorical			
Units: Subjects			
Female			
Male			
Ethnicity (NIH/OMB)			
Units: Subjects			
Hispanic or Latino			
Not Hispanic or Latino			
Unknown or Not Reported			
Race (NIH/OMB)			
Units: Subjects			
American Indian or Alaska Native			
Asian			
Native Hawaiian or Other Pacific Islander			
Black or African American			
White			
More than one race			
Unknown or Not Reported			
Weight			
Units: kilogram (kg)			
arithmetic mean			
standard deviation	±	±	±
Height			
Units: centimeter (cm)			
arithmetic mean			
standard deviation	±	±	±
Body Mass Index (BMI)			
BMI is calculated as [weight (kg) / height(m)^2]			
Units: kg/m^2			
arithmetic mean			
standard deviation	±	±	±

Reporting group values	Age 2-7 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg	Age 8-13years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg	Age 2-11years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg

Number of subjects	2	4	4
Age Categorical Units: Subjects			

Age continuous Units: years arithmetic mean standard deviation	± ±	± ±	± ±
Gender categorical Units: Subjects			
Female Male			
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino Not Hispanic or Latino Unknown or Not Reported			
Race (NIH/OMB) Units: Subjects			
American Indian or Alaska Native Asian Native Hawaiian or Other Pacific Islander Black or African American White More than one race Unknown or Not Reported			
Weight Units: kilogram (kg) arithmetic mean standard deviation	± ±	± ±	± ±
Height Units: centimeter (cm) arithmetic mean standard deviation	± ±	± ±	± ±
Body Mass Index (BMI)			
BMI is calculated as [weight (kg) / height(m)^2]			
Units: kg/m^2 arithmetic mean standard deviation	± ±	± ±	± ±

Reporting group values	Age ≥12 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg	Age 2-12years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg	Age ≥13 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Number of subjects	13	6	11
Age Categorical Units: Subjects			

Age continuous Units: years arithmetic mean standard deviation	± ±	± ±	± ±
Gender categorical Units: Subjects			
Female Male			
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino Not Hispanic or Latino Unknown or Not Reported			
Race (NIH/OMB) Units: Subjects			
American Indian or Alaska Native Asian Native Hawaiian or Other Pacific Islander Black or African American White More than one race Unknown or Not Reported			
Weight Units: kilogram (kg) arithmetic mean standard deviation	± ±	± ±	± ±
Height Units: centimeter (cm) arithmetic mean standard deviation	± ±	± ±	± ±
Body Mass Index (BMI)			
BMI is calculated as [weight (kg) / height(m)^2]			
Units: kg/m^2 arithmetic mean standard deviation	± ±	± ±	± ±

Reporting group values	Age 2-13years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg	Age ≥14 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg	IGSC: Epoch 2: 2-13 years
Number of subjects	6	11	4
Age Categorical Units: Subjects			

Age continuous Units: years arithmetic mean standard deviation	± ±	± ±	± ±

Gender categorical Units: Subjects			
Female Male			
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino Not Hispanic or Latino Unknown or Not Reported			
Race (NIH/OMB) Units: Subjects			
American Indian or Alaska Native Asian Native Hawaiian or Other Pacific Islander Black or African American White More than one race Unknown or Not Reported			
Weight Units: kilogram (kg) arithmetic mean standard deviation	±	±	±
Height Units: centimeter (cm) arithmetic mean standard deviation	±	±	±
Body Mass Index (BMI)			
BMI is calculated as [weight (kg) / height(m)^2]			
Units: kg/m^2 arithmetic mean standard deviation	±	±	±

Reporting group values	IGSC: Epoch 2: >=14 years	IGSC: Epoch 3: 2-13 years	IGSC: Epoch 3: >=14 years
Number of subjects	6	2	7
Age Categorical Units: Subjects			
Age continuous Units: years arithmetic mean standard deviation	±	±	±
Gender categorical Units: Subjects			
Female Male			
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino Not Hispanic or Latino Unknown or Not Reported			

Race (NIH/OMB) Units: Subjects			
American Indian or Alaska Native Asian Native Hawaiian or Other Pacific Islander Black or African American White More than one race Unknown or Not Reported			
Weight Units: kilogram (kg) arithmetic mean standard deviation	±	±	±
Height Units: centimeter (cm) arithmetic mean standard deviation	±	±	±
Body Mass Index (BMI)			
BMI is calculated as [weight (kg) / height(m)^2]			
Units: kg/m^2 arithmetic mean standard deviation	±	±	±

Reporting group values	IGIV: Epoch 1: 3-week interval	IGIV: Epoch 1: 4-week interval	IGSC: Epoch 3: Biweekly
Number of subjects	17	17	7
Age Categorical Units: Subjects			
Age continuous Units: years arithmetic mean standard deviation	±	±	±
Gender categorical Units: Subjects			
Female Male			
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino Not Hispanic or Latino Unknown or Not Reported			
Race (NIH/OMB) Units: Subjects			
American Indian or Alaska Native Asian Native Hawaiian or Other Pacific Islander Black or African American White More than one race Unknown or Not Reported			

Weight			
Units: kilogram (kg)			
arithmetic mean			
standard deviation	±	±	±
Height			
Units: centimeter (cm)			
arithmetic mean			
standard deviation	±	±	±
Body Mass Index (BMI)			
BMI is calculated as [weight (kg) / height(m)^2]			
Units: kg/m^2			
arithmetic mean			
standard deviation	±	±	±

Reporting group values	IGSC: Epoch 2	IGIV 200 to 600 mg/kg + IGSC [50 to 200 and 100 to 400] mg/kg	Epoch 2: IGSC (20%) 50-200 mg/kg
Number of subjects	17	17	17
Age Categorical			
Units: Subjects			
Age continuous			
Units: years			
arithmetic mean		31.0	
standard deviation	±	± 21.13	±
Gender categorical			
Units: Subjects			
Female		7	0
Male		10	0
Ethnicity (NIH/OMB)			
Units: Subjects			
Hispanic or Latino		0	0
Not Hispanic or Latino		17	0
Unknown or Not Reported		0	0
Race (NIH/OMB)			
Units: Subjects			
American Indian or Alaska Native		0	0
Asian		17	0
Native Hawaiian or Other Pacific Islander		0	0
Black or African American		0	0
White		0	0
More than one race		0	0
Unknown or Not Reported		0	0
Weight			
Units: kilogram (kg)			
arithmetic mean		48.16	0
standard deviation	±	± 18.120	±
Height			
Units: centimeter (cm)			
arithmetic mean		152.15	0
standard deviation	±	± 20.076	±

Body Mass Index (BMI)			
BMI is calculated as [weight (kg) / height(m)^2]			
Units: kg/m^2			
arithmetic mean		19.88	0
standard deviation	±	± 3.242	±

Reporting group values	Epoch 3: IGSC (20%) 100-400 mg/kg	Epoch 1: IGIV 3-week Interval	Epoch 1: IGIV 4-week Interval
Number of subjects	7	5	12
Age Categorical			
Units: Subjects			
Age continuous			
Units: years			
arithmetic mean			
standard deviation	±	±	±
Gender categorical			
Units: Subjects			
Female	0	0	0
Male	0	0	0
Ethnicity (NIH/OMB)			
Units: Subjects			
Hispanic or Latino	0	0	0
Not Hispanic or Latino	0	0	0
Unknown or Not Reported	0	0	0
Race (NIH/OMB)			
Units: Subjects			
American Indian or Alaska Native	0	0	0
Asian	0	0	0
Native Hawaiian or Other Pacific Islander	0	0	0
Black or African American	0	0	0
White	0	0	0
More than one race	0	0	0
Unknown or Not Reported	0	0	0
Weight			
Units: kilogram (kg)			
arithmetic mean	0	0	0
standard deviation	±	±	±
Height			
Units: centimeter (cm)			
arithmetic mean	0	0	0
standard deviation	±	±	±
Body Mass Index (BMI)			
BMI is calculated as [weight (kg) / height(m)^2]			
Units: kg/m^2			
arithmetic mean	0	0	0
standard deviation	±	±	±

Reporting group values	Epoch 2: IGSC, 20% Weekly	Epoch 2: IGSC, 20% Weekly (EOS [Week 25]/ ET)	Epoch 3: IGSC, 20% Biweekly

Number of subjects	17	10	7
Age Categorical Units: Subjects			

Age continuous Units: years arithmetic mean standard deviation	±	±	±
Gender categorical Units: Subjects			
Female	0	0	0
Male	0	0	0
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino	0	0	0
Not Hispanic or Latino	0	0	0
Unknown or Not Reported	0	0	0
Race (NIH/OMB) Units: Subjects			
American Indian or Alaska Native	0	0	0
Asian	0	0	0
Native Hawaiian or Other Pacific Islander	0	0	0
Black or African American	0	0	0
White	0	0	0
More than one race	0	0	0
Unknown or Not Reported	0	0	0
Weight Units: kilogram (kg) arithmetic mean standard deviation	0 ±	0 ±	0 ±
Height Units: centimeter (cm) arithmetic mean standard deviation	0 ±	0 ±	0 ±
Body Mass Index (BMI)			
BMI is calculated as [weight (kg) / height(m)^2]			
Units: kg/m^2 arithmetic mean standard deviation	0 ±	0 ±	0 ±

Reporting group values	Epoch 3: IGSC, 20% Biweekly (Week 13)		
Number of subjects	7		
Age Categorical Units: Subjects			

Age continuous Units: years arithmetic mean standard deviation	±		

Gender categorical Units: Subjects			
Female	0		
Male	0		
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino	0		
Not Hispanic or Latino	0		
Unknown or Not Reported	0		
Race (NIH/OMB) Units: Subjects			
American Indian or Alaska Native	0		
Asian	0		
Native Hawaiian or Other Pacific Islander	0		
Black or African American	0		
White	0		
More than one race	0		
Unknown or Not Reported	0		
Weight Units: kilogram (kg)			
arithmetic mean	0		
standard deviation	±		
Height Units: centimeter (cm)			
arithmetic mean	0		
standard deviation	±		
Body Mass Index (BMI)			
BMI is calculated as [weight (kg) / height(m)^2]			
Units: kg/m^2			
arithmetic mean	0		
standard deviation	±		

End points

End points reporting groups

Reporting group title	Epoch 1: IGIV 200-600 mg/kg
Reporting group description:	
Participants received 200 to 600 mg/kg of Immunoglobulin Intravenous (IGIV) infusion for every 3 or 4 weeks for up to 13 weeks.	
Reporting group title	Epoch 2: IGSC (20%) 50-200 mg/kg
Reporting group description:	
Participants who entered to Epoch 2 from Epoch 1 received 50-200 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion once a week up to approximately 24 weeks after Epoch 1.	
Reporting group title	Epoch 3: IGSC (20%) 100-400 mg/kg
Reporting group description:	
Participants who entered to Epoch 3 from Epoch 2 received 100-400 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion biweekly up to approximately 12 weeks after Epoch 2.	
Subject analysis set title	Epoch 1: Immune Globulin Intravenous (IGIV)
Subject analysis set type	Full analysis
Subject analysis set description:	
Participants received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for up to 13 weeks.	
Subject analysis set title	Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC)
Subject analysis set type	Full analysis
Subject analysis set description:	
Participants who entered to Epoch 2 from Epoch 1 received 50-200 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion once a week up to approximately 24 weeks after Epoch 1.	
Subject analysis set title	Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Subject analysis set type	Full analysis
Subject analysis set description:	
Participants who entered to Epoch 3 from Epoch 2 received 100-400 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion biweekly up to approximately 12 weeks after Epoch 2.	
Subject analysis set title	Age 2-7 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Sub-group analysis
Subject analysis set description:	
Participants aged 2-7 years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.	
Subject analysis set title	Age 8-13years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Sub-group analysis
Subject analysis set description:	
Participants aged 8-13 years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.	
Subject analysis set title	Age 2-11years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Full analysis
Subject analysis set description:	
Participants aged 2-11 years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.	
Subject analysis set title	Age ≥12 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Full analysis

Subject analysis set description:

Participants aged >=12 years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	Age 2-12years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Full analysis

Subject analysis set description:

Participants aged 2-12 years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	Age ≥13 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Full analysis

Subject analysis set description:

Participants aged >=13 years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	Age 2-13years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Full analysis

Subject analysis set description:

Participants aged 2-13 years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	Age ≥14 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-400]mg/kg
Subject analysis set type	Full analysis

Subject analysis set description:

Participants aged 14 and above years received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks for a total of 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	IGSC: Epoch 2: 2-13 years
Subject analysis set type	Sub-group analysis

Subject analysis set description:

Participants aged 2-13 years received approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch2.

Subject analysis set title	IGSC: Epoch 2: >=14 years
Subject analysis set type	Sub-group analysis

Subject analysis set description:

Participants aged >=14 years received approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2.

Subject analysis set title	IGSC: Epoch 3: 2-13 years
Subject analysis set type	Sub-group analysis

Subject analysis set description:

Participants aged 2-13 years received approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	IGSC: Epoch 3: >=14 years
Subject analysis set type	Sub-group analysis

Subject analysis set description:

Participants aged >=14 years received approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	IGIV: Epoch 1: 3-week interval
Subject analysis set type	Full analysis

Subject analysis set description:

Participants received approximately 200 to 600 mg/kg of IGIV infusion for every 3 for a total of 13 weeks.

Subject analysis set title	IGIV: Epoch 1: 4-week interval
Subject analysis set type	Full analysis

Subject analysis set description:

Participants received approximately 200 to 600 mg/kg of IGIV infusion for every 4 for a total of 13 weeks.

Subject analysis set title	IGSC: Epoch 3: Biweekly
Subject analysis set type	Full analysis

Subject analysis set description:

Participants received approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 1.

Subject analysis set title	IGSC: Epoch 2
Subject analysis set type	Full analysis

Subject analysis set description:

Participants received approximately 50 to 200 mg/kg of IGSC infusion, 20% once a week for a total of 24 weeks after Epoch 1.

Subject analysis set title	IGIV 200 to 600 mg/kg + IGSC [50 to 200 and 100 to 400] mg/kg
Subject analysis set type	Full analysis

Subject analysis set description:

Participants received 200 to 600 mg/kg of IGIV infusion for every 3 or 4 weeks up to 13 weeks in Epoch 1; followed by approximately 50 to 200 mg/kg of IGSC infusion,20% once a week for a total of 24 weeks after Epoch 1 in Epoch 2; followed by approximately 100 to 400 mg/kg of IGSC infusion, 20% once every two weeks for a total of 12 weeks after Epoch 2 in Epoch 3.

Subject analysis set title	Epoch 2: IGSC (20%) 50-200 mg/kg
Subject analysis set type	Full analysis

Subject analysis set description:

Participants who entered to Epoch 2 from Epoch 1 received 50-200 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion once a week up to approximately 24 weeks after Epoch 1.

Subject analysis set title	Epoch 3: IGSC (20%) 100-400 mg/kg
Subject analysis set type	Full analysis

Subject analysis set description:

Participants who entered to Epoch 3 from Epoch 2 received 100-400 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion biweekly up to approximately 12 weeks after Epoch 2.

Subject analysis set title	Epoch 1: IGIV 3-week Interval
Subject analysis set type	Full analysis

Subject analysis set description:

Participants received 200 to 600 mg/kg of Immunoglobulin Intravenous (IGIV) infusion for every 3 weeks for up to 13 weeks.

Subject analysis set title	Epoch 1: IGIV 4-week Interval
Subject analysis set type	Full analysis

Subject analysis set description:

Participants received 200 to 600 mg/kg of Immunoglobulin Intravenous (IGIV) infusion for every 4 weeks for up to 13 weeks.

Subject analysis set title	Epoch 2: IGSC, 20% Weekly
Subject analysis set type	Full analysis

Subject analysis set description:

Participants who entered to Epoch 2 from Epoch 1 received 50-200 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion once a week up to approximately 24 weeks after Epoch 1.

Subject analysis set title	Epoch 2: IGSC, 20% Weekly (EOS [Week 25]/ ET)
Subject analysis set type	Full analysis

Subject analysis set description:

Participants who entered to Epoch 2 from Epoch 1 received 50-200 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion once a week up to end of study (EOS) (24 weeks) or early

termination (ET) after Epoch 1.

Subject analysis set title	Epoch 3: IGSC, 20% Biweekly
Subject analysis set type	Full analysis

Subject analysis set description:

Participants who entered to Epoch 3 from Epoch 2 received 100-400 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion biweekly up to EOS (13 weeks) after Epoch 2.

Subject analysis set title	Epoch 3: IGSC, 20% Biweekly (Week 13)
Subject analysis set type	Full analysis

Subject analysis set description:

Participants who entered to Epoch 3 from Epoch 2 received 100-400 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion biweekly up to EOS (13 weeks) after Epoch 2.

Primary: Epoch 2: Total Serum Trough Levels of Immune Globulin G (IgG) Antibodies During Period 2

End point title	Epoch 2: Total Serum Trough Levels of Immune Globulin G (IgG) Antibodies During Period 2[1]

End point description:

Total serum trough levels of IgG antibodies measured during period 2 of Epoch 2 were assessed. Pharmacokinetic Analysis Set (PKAS) 1 included all participants in the all-treated set who have had at least 1 evaluable serum IgG concentration value and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Only PKAS 1 participants in Epoch 2 were analyzed for this outcome measure.Total serum trough levels of IgG antibodies measured during period 2 of Epoch 2 were assessed.

End point type	Primary

End point timeframe:

Epoch 2 (period 2): Up to 24 weeks

Notes:

[1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: No statistical analyses have been specified for this primary end point.

End point values	Epoch 2: IGSC (20%) 50-200 mg/kg			
Subject group type	Reporting group			
Number of subjects analysed	17			
Units: grams per liter (g/L)				
geometric mean (confidence interval 95%)	8.56 (8.03 to 9.12)			

Statistical analyses

No statistical analyses for this end point

Primary: Epoch 3: Total Serum Trough Levels of IgG Antibodies

End point title	Epoch 3: Total Serum Trough Levels of IgG Antibodies[2]

End point description:

Total serum trough levels of IgG antibodies measured during Epoch 3 were assessed. PKAS 1 included all participants in the all-treated set who have had at least 1 evaluable serum IgG concentration value and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Only PKAS 1 participants in Epoch 3 were analyzed for this outcome measure.

End point type	Primary

End point timeframe:

Epoch 3: Up to Week 12

Notes:

[2] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: No statistical analyses have been specified for this primary end point.

End point values	Epoch 3: IGSC (20%) 100-400 mg/kg			
Subject group type	Reporting group			
Number of subjects analysed	7			
Units: g/L				
geometric mean (confidence interval 95%)	8.39 (7.89 to 8.91)			

Statistical analyses

No statistical analyses for this end point

Secondary: Epoch 1: Total Serum Trough Levels of IgG Antibodies

End point title	Epoch 1: Total Serum Trough Levels of IgG Antibodies

End point description:

Total serum trough levels of IgG antibodies measured during Epoch 1 were assessed. PKAS 1 included all participants in the all-treated set who have had at least 1 evaluable serum IgG concentration value and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Only PKAS 1 participants in Epoch 1 were analyzed for this outcome measure.

End point type	Secondary

End point timeframe:

Epoch 1: Up to Week 13

End point values	Epoch 1: IGIV 200-600 mg/kg			
Subject group type	Reporting group			
Number of subjects analysed	17			
Units: g/L				
geometric mean (confidence interval 95%)	8.18 (7.71 to 8.68)			

Statistical analyses

No statistical analyses for this end point

Secondary: Epoch 2: Area Under the Curve From Time 0 to Last Interval (AUC0-last) for Total Serum Levels of IgG

End point title	Epoch 2: Area Under the Curve From Time 0 to Last Interval (AUC0-last) for Total Serum Levels of IgG

End point description:

PKAS 2 included all participants >=12 years of age in the all-treated set who have had at least 1

evaluable serum IgG concentration value during Epoch 2 and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Overall number analyzed are the number of participants with data available for analyses.

End point type	Secondary

End point timeframe:

Epoch 2: Week 21

End point values	Epoch 2: IGSC (20%) 50-200 mg/kg			
Subject group type	Reporting group			
Number of subjects analysed	7			
Units: g*day/L				
geometric mean (confidence interval 95%)	58.9 (48.7 to 71.3)			

Statistical analyses

No statistical analyses for this end point

Secondary: Epoch 2: AUC0-last for Total Serum Levels of IgG Subclasses

End point title	Epoch 2: AUC0-last for Total Serum Levels of IgG Subclasses

End point description:

Total serum levels of IgG subclasses IgG 1, IgG 2, IgG 3, and IgG 4 were determined. PKAS 2 included all participants >=12 years of age in the all-treated set who have had at least 1 evaluable serum IgG concentration value during Epoch 2 and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Overall number analyzed are the number of participants with data available for analyses.

End point type	Secondary

End point timeframe:

Epoch 2: Week 21

End point values	Epoch 2: IGSC (20%) 50-200 mg/kg			
Subject group type	Reporting group			
Number of subjects analysed	7			
Units: g*day/L				
geometric mean (confidence interval 95%)				
IgG 1	35.8 (32.4 to 39.5)			
IgG 2	23.4 (20.4 to 26.9)			
IgG 3	1.32 (0.624 to 2.80)			
IgG 4	1.67 (1.10 to 2.53)			

Statistical analyses

No statistical analyses for this end point

Secondary: Epoch 2: Apparent Clearance (CL/F) for Total Serum Levels of IgG

End point title	Epoch 2: Apparent Clearance (CL/F) for Total Serum Levels of IgG

End point description:

PKAS 2 included all participants >=12 years of age in the all-treated set who have had at least 1 evaluable serum IgG concentration value during Epoch 2 and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Overall number analyzed are the number of participants with data available for analyses.

End point type	Secondary

End point timeframe:

Epoch 2: Week 21

End point values	Epoch 2: IGSC (20%) 50-200 mg/kg			
Subject group type	Reporting group			
Number of subjects analysed	7			
Units: mL/kg/day				
geometric mean (confidence interval 95%)	1.93 (1.71 to 2.19)			

Statistical analyses

No statistical analyses for this end point

Secondary: Epoch 2: CL/F for Total Serum Levels of IgG Subclasses

End point title	Epoch 2: CL/F for Total Serum Levels of IgG Subclasses

End point description:

PKAS 2 included all participants >=12 years of age in the all-treated set who have had at least 1 evaluable serum IgG concentration value during Epoch 2 and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Overall number analyzed are the number of participants with data available for analyses.

End point type	Secondary

End point timeframe:

Epoch 2: Week 21

End point values	Epoch 2: IGSC (20%) 50-200 mg/kg			
Subject group type	Reporting group			
Number of subjects analysed	7			
Units: mL/kg/day				
geometric mean (confidence interval 95%)				
IgG 1	3.18 (2.88 to 3.51)			
IgG 2	4.86 (4.18 to 5.66)			
IgG 3	99.5 (37.1 to 266)			
IgG 4	68.3 (48.3 to 96.6)			

Statistical analyses

No statistical analyses for this end point

Secondary: Epoch 2: Maximum Concentration (Cmax) for Total Serum Levels of IgG

End point title	Epoch 2: Maximum Concentration (Cmax) for Total Serum Levels of IgG

End point description:

PKAS 2 included all participants >=12 years of age in the all-treated set who have had at least 1 evaluable serum IgG concentration value during Epoch 2 and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Overall number analyzed are the number of participants with data available for analyses.

End point type	Secondary

End point timeframe:

Epoch 2: Week 21

End point values	Epoch 2: IGSC (20%) 50-200 mg/kg			
Subject group type	Reporting group			
Number of subjects analysed	7			
Units: g/L				
geometric mean (confidence interval 95%)	9.08 (7.46 to 11.1)			

Statistical analyses

No statistical analyses for this end point

Secondary: Epoch 2: Cmax for Total Serum Levels of IgG Subclasses

End point title	Epoch 2: Cmax for Total Serum Levels of IgG Subclasses

End point description:

Total serum levels of IgG subclasses IgG 1, IgG 2, IgG 3, and IgG 4 were determined. PKAS 2 included all participants >=12 years of age in the all-treated set who have had at least 1 evaluable serum IgG concentration value during Epoch 2 and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Overall number analyzed are the number of participants with data available for analyses.

End point type	Secondary

End point timeframe:

Epoch 2: Week 21

End point values	Epoch 2: IGSC (20%) 50-200 mg/kg			
Subject group type	Reporting group			
Number of subjects analysed	7			
Units: g/L				
geometric mean (confidence interval 95%)				
IgG 1	5.40 (4.95 to 5.89)			
IgG 2	3.51 (3.06 to 4.03)			
IgG 3	0.202 (0.0960 to 0.425)			
IgG 4	0.256 (0.169 to 0.387)			

Statistical analyses

No statistical analyses for this end point

Secondary: Epoch 2: Minimum Concentration (Cmin) for Total Serum Levels of IgG

End point title	Epoch 2: Minimum Concentration (Cmin) for Total Serum Levels of IgG

End point description:

PKAS 2 included all participants >=12 years of age in the all-treated set who have had at least 1 evaluable serum IgG concentration value during Epoch 2 and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Overall number analyzed are the number of participants with data available for analyses.

End point type	Secondary

End point timeframe:

Epoch 2: Week 21

End point values	Epoch 2: IGSC (20%) 50-200 mg/kg			
Subject group type	Reporting group			
Number of subjects analysed	7			
Units: g/L				
geometric mean (confidence interval 95%)	7.45 (6.17 to 8.99)			

Statistical analyses

No statistical analyses for this end point

Secondary: Epoch 2: Cmin for Total Serum Levels of IgG Subclasses

End point title	Epoch 2: Cmin for Total Serum Levels of IgG Subclasses

End point description:

Total serum levels of IgG subclasses IgG 1, IgG 2, IgG 3, and IgG 4 were determined. PKAS 2 included all participants >=12 years of age in the all-treated set who have had at least 1 evaluable serum IgG concentration value during Epoch 2 and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Overall number analyzed are the number of participants with data available for analyses.

End point type	Secondary

End point timeframe:

Epoch 2: Week 21

End point values	Epoch 2: IGSC (20%) 50-200 mg/kg			
Subject group type	Reporting group			
Number of subjects analysed	7			
Units: g/L				
geometric mean (confidence interval 95%)				
IgG 1	4.89 (4.34 to 5.51)			
IgG 2	3.15 (2.70 to 3.68)			
IgG 3	0.181 (0.0861 to 0.381)			
IgG 4	0.218 (0.153 to 0.311)			

Statistical analyses

No statistical analyses for this end point

Secondary: Epoch 2: Time to Maximum Concentration (Tmax) for Total Serum Levels of IgG

End point title	Epoch 2: Time to Maximum Concentration (Tmax) for Total Serum Levels of IgG

End point description:

PKAS 2 included all participants >=12 years of age in the all-treated set who have had at least 1 evaluable serum IgG concentration value during Epoch 2 and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Overall number analyzed are the number of participants with data available for analyses.

End point type	Secondary

End point timeframe:

Epoch 2: Week 21

End point values	Epoch 2: IGSC (20%) 50-200 mg/kg			
Subject group type	Reporting group			
Number of subjects analysed	7			
Units: hours				
median (full range (min-max))	71.08 (22.82 to 168.23)			

Statistical analyses

No statistical analyses for this end point

Secondary: Epoch 2: Tmax for Total Serum Levels of IgG Subclasses

End point title	Epoch 2: Tmax for Total Serum Levels of IgG Subclasses

End point description:

Total serum levels of IgG subclasses IgG 1, IgG 2, IgG 3, and IgG 4 were determined. PKAS 2 included all participants >=12 years of age in the all-treated set who have had at least 1 evaluable serum IgG concentration value during Epoch 2 and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Overall number analyzed are the number of participants with data available for analyses.

End point type	Secondary

End point timeframe:

Epoch 2: Week 21

End point values	Epoch 2: IGSC (20%) 50-200 mg/kg			
Subject group type	Reporting group			
Number of subjects analysed	7			
Units: hours				
median (full range (min-max))				
IgG 1	71.08 (22.82 to 118.80)			
IgG 2	71.08 (0.00 to 164.32)			

IgG 3 IgG 4	72.92 (71.08 to 118.80) 71.17 (70.13 to 118.80)			

Statistical analyses

No statistical analyses for this end point

Secondary: Trough Levels of Specific Antibodies to Clinically Relevant Pathogens: Clostridium Tetani Toxoid and Hepatitis B Virus (HBV)

End point title	Trough Levels of Specific Antibodies to Clinically Relevant Pathogens: Clostridium Tetani Toxoid and Hepatitis B Virus (HBV)

End point description:

Trough levels of specific antibodies to clinically relevant pathogen (Clostridium tetani toxoid and HBV) were assessed in Epoch 1, Epoch 2 and Epoch 3. Data was analyzed per interval in each Epoch for this outcome measure. PKAS 1 included all participants in the all-treated set who have had at least 1 evaluable serum IgG concentration value and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Overall number analyzed are the number of participants available for analyses. Data is represented as per doses received for specific interval in each Epoch for this outcome measure.

End point type	Secondary

End point timeframe:

Epoch 1 (Week 1); Epoch 2 (Week 1, 24); Epoch 3 (Week 1, 13)

End point values	Epoch 1: IGIV 3-week Interval	Epoch 1: IGIV 4-week Interval	Epoch 2: IGSC, 20% Weekly	Epoch 2: IGSC, 20% Weekly (EOS [Week 25]/ ET)
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	5	12	17	10
Units: International units (IU)/mL				
geometric mean (confidence interval 95%)				
Clostridium Tetani Toxoid	0.524 (0.290 to 0.949)	0.602 (0.364 to 0.997)	0.674 (0.468 to 0.970)	1.52 (1.11 to 2.08)
Hepatitis B virus	22.7 (17.8 to 43.0)	35.2 (17.5 to 70.9)	54.1 (33.7 to 87.0)	486 (335 to 706)

End point values	Epoch 3: IGSC, 20% Biweekly	Epoch 3: IGSC, 20% Biweekly (Week 13)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	7	7		
Units: International units (IU)/mL				
geometric mean (confidence interval 95%)				
Clostridium Tetani Toxoid	1.97 (1.65 to 2.35)	1.80 (1.35 to 2.39)		

Hepatitis B virus	645 (452 to 920)	923 (832 to 1020)		

Statistical analyses

No statistical analyses for this end point

Secondary: Trough Levels of Specific Antibodies to Clinically Relevant Pathogen: Haemophilus Influenzae (HIB)

End point title	Trough Levels of Specific Antibodies to Clinically Relevant Pathogen: Haemophilus Influenzae (HIB)

End point description:

Trough levels of specific antibodies to clinically relevant pathogens (HIB) were assessed in Epoch 1, Epoch 2 and Epoch 3. Data was analyzed per interval in each Epoch for this outcome measure. PKAS 1 included all participants in the all-treated set who have had at least 1 evaluable serum IgG concentration value and have had no major protocol deviations or events that would affect the serum IgG concentration analysis results. Overall number analyzed are the number of participants available for analyses. Data is represented as per doses received for specific intervals in each Epoch for this outcome measure.

End point type	Secondary

End point timeframe:

Epoch 1 (Week 1); Epoch 2 (Week 1, 24); Epoch 3 (Week 1, 13)

End point values	Epoch 1: IGIV 3-week Interval	Epoch 1: IGIV 4-week Interval	Epoch 2: IGSC, 20% Weekly	Epoch 2: IGSC, 20% Weekly (EOS [Week 25]/ ET)
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	5	12	17	10
Units: mg/L				
geometric mean (confidence interval 95%)	1.58 (0.568 to 4.38)	1.55 (1.11 to 2.18)	2.50 (1.95 to 3.20)	1.77 (1.07 to 2.91)

End point values	Epoch 3: IGSC, 20% Biweekly	Epoch 3: IGSC, 20% Biweekly (Week 13)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	7	7		
Units: mg/L				
geometric mean (confidence interval 95%)	2.04 (1.74 to 2.39)	1.73 (1.46 to 2.04)		

Statistical analyses

No statistical analyses for this end point

Secondary: Health Related Quality of Life: Treatment Preference

End point title	Health Related Quality of Life: Treatment Preference

End point description:

Treatment preference questionnaire is self-administered questionnaire developed to assess participants' preference towards administration of new IGSC therapy. There are 4-items on questionnaire, which investigate participant's preference on clinic/hospital/home setting of receiving the immunoglobulin (Ig) therapy, the participant's rating on the frequency and method of administration, and the participant's preference to continue receiving the IGSC treatment. The questionnaire included following categories: Q1=Where do you prefer to receive your immunoglobulin therapy, Q2=The Frequency of Administration: Liked Very Much, Q3=The Frequency of Administration: Liked. As pre-specified in protocol, data is reported per age (2-13 and >=14 years). All-Treated Set=all enrolled participants of age '2-13 years' and '>=14 years' who received at least 1 dose of study drug (IGIV or IGSC). 'n' = Number analyzed is the number of participants with data available for analyses at specific timepoints.

End point type	Secondary

End point timeframe:

Up to approximately 1.5 years

End point values	IGSC: Epoch 2: 2-13 years	IGSC: Epoch 2: >=14 years	IGSC: Epoch 3: 2-13 years	IGSC: Epoch 3: >=14 years
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	4	6	2	5
Units: participants				
Where do you Prefer to Receive Ig Therapy-At Home	3	1	1	4
The Frequency of Administration: Liked Very Much	2	1	1	4
The Frequency of Administration: Liked	1	3	1	1

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Participants with Treatment Emergent Adverse Events (TEAEs)

End point title	Number of Participants with Treatment Emergent Adverse Events (TEAEs)

End point description:

TEAEs was defined as adverse events (AEs) with onset after date-time of first dose of study drug, or medical conditions present prior to the start of IP but increased in severity or relationship after date-time of first dose of IP. Any TEAE that is recorded by the investigator as "possibly related" or "probably related" to IP was considered as IGSC, 20%-related AE, and any AE recorded as "unlikely related" or "not related" was considered as unrelated AE. AEs included vital signs, clinical laboratory measurements. All-Treated Set included all enrolled participants who received at least 1 dose of study drug (IGIV or IGSC).

End point type	Secondary

End point timeframe:

From first dose of study drug up to end of study (up to approximately 1.5 years)

End point values	Epoch 1: Immune Globulin Intravenous (IGIV)	Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC)	Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)	
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	
Number of subjects analysed	17	17	7	
Units: participants	11	16	3	

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Participants with Tolerability Events Related to the Infusion of Study Drug

End point title	Number of Participants with Tolerability Events Related to the Infusion of Study Drug

End point description:

An infusion is considered tolerable if the infusion rate was not reduced, or the infusion was not interrupted or stopped, due to TEAE related to study drug (IGIV or IGSC) infusion. A tolerability event is considered to have occurred if an infusion was not tolerable in Epoch 1, Epoch 2 and Epoch 3. Number of participants with tolerability events related to infusion of IP were assessed. All-Treated Set included all enrolled participants who received at least 1 dose of study drug (IGIV or IGSC).

End point type	Secondary

End point timeframe:

From first dose of study drug up to end of study (up to approximately 1.5 years)

End point values	Epoch 1: Immune Globulin Intravenous (IGIV)	Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC)	Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)	
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	
Number of subjects analysed	17	17	7	
Units: participants				
Infusions With Infusion Rate Reduced	0	0	0	
Infusions That Were Interrupted	0	1	0	
Infusions That Were Stopped	0	0	0	
Infusion Rate Reduced/Interrupted/Stopped	0	1	0	

Statistical analyses

No statistical analyses for this end point

Secondary: Annual Rate of Validated Acute Serious Bacterial Infections (ASBI)

End point title	Annual Rate of Validated Acute Serious Bacterial Infections (ASBI)

End point description:

The ASBI rate was calculated as the mean number of acute serious bacterial infections per participants per year. Annual rate of validated acute serious bacterial infections per participant was assessed. All-Treated Set included all enrolled participants who received at least 1 dose of study drug (IGIV or IGSC).

End point type	Secondary

End point timeframe:

From first dose of study drug up to end of study (up to approximately 1.5 years)

End point values	Epoch 1: Immune Globulin Intravenous (IGIV)	Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC)	Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)	
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	
Number of subjects analysed	17	17	7	
Units: number of infections per year				
arithmetic mean (standard deviation)	0.0 (± 0.00)	0.25 (± 1.042)	0.0 (± 0.00)	

Statistical analyses

No statistical analyses for this end point

Secondary: Annual Rate of All Infections Per Year

End point title	Annual Rate of All Infections Per Year

End point description:

Annual rate is the number of participants reporting any infection per year. All-Treated Set included all enrolled participants who received at least 1 dose of study drug (IGIV or IGSC).

End point type	Secondary

End point timeframe:

From first dose of study drug up to end of study (up to approximately 1.5 years)

End point values	Epoch 1: Immune Globulin Intravenous (IGIV)	Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC)	Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)	
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	
Number of subjects analysed	17	17	7	
Units: number of infections per year				
arithmetic mean (standard deviation)	1.65 (± 2.479)	2.78 (± 3.074)	0.00 (± 0.000)	

Statistical analyses

Statistical analysis title	Epoch 1: IGIV
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	1.65
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.73
upper limit	3.15

Statistical analysis title	Epoch 3: IGSC
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	0
Confidence interval	
level	95 %
sides	2-sided
lower limit	0
upper limit	0

Statistical analysis title	Epoch 2: IGSC
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)

Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	2.48
Confidence interval	
level	95 %
sides	2-sided
lower limit	1.34
upper limit	4.13

Secondary: Number of Days Participants not Able to Attend School or Work to Perform Normal Daily Activities due to Illness/Infection

End point title	Number of Days Participants not Able to Attend School or Work to Perform Normal Daily Activities due to Illness/Infection

End point description:

Number of days not able to attend school or work to perform normal daily activities due to illness/infection are standardized per year (365.25 days). The number of days not able to attend school or work to perform normal daily activities due to illness/infection were assessed. All-Treated Set included all enrolled participants who received at least 1 dose of study drug (IGIV or IGSC).

End point type	Secondary

End point timeframe:

From first dose of study drug up to end of study (up to approximately 1.5 years)

End point values	Epoch 1: Immune Globulin Intravenous (IGIV)	Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC)	Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)	
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	
Number of subjects analysed	17	17	7	
Units: days				
median (full range (min-max))	0.00 (0.0 to 16.1)	0.00 (0.0 to 17.2)	4.30 (0.0 to 38.7)	

Statistical analyses

Statistical analysis title	Epoch 1: IGIV
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)

Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	2.6
Confidence interval	
level	95 %
sides	2-sided
lower limit	1.02
upper limit	5.3

Statistical analysis title	Epoch 2: IGSC
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	2.87
Confidence interval	
level	95 %
sides	2-sided
lower limit	1.37
upper limit	5.18

Statistical analysis title	Epoch 3: IGSC
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	9.82
Confidence interval	
level	95 %
sides	2-sided
lower limit	2.82
upper limit	23.82

Secondary: Number of Days Participants Were on Antibiotics

End point title	Number of Days Participants Were on Antibiotics

End point description:

Number of days on antibiotics is defined as the number of days those antibiotics were taken as

concomitant medications and is standardized to per year (365.25 days). Antibiotics are defined as any medication under anatomical therapeutic chemical Level 2 therapeutic class “ANTIBACTERIALS FOR SYSTEMIC USE”. If a participant took multiple antibiotics on a single day, that day is counted for only once. Protocol defined prophylactic antibiotics for viral, fungal or protozoal infections (e.g. trimethoprim/sulfamethoxazole twice a week for pneumocystis) which are not treated by immunoglobulin, were excluded from this analysis. All-Treated Set included all enrolled participants who received at least 1 dose of study drug (IGIV or IGSC).

End point type	Secondary

End point timeframe:

From first dose of study drug up to end of study (up to approximately 1.5 years)

End point values	Epoch 1: Immune Globulin Intravenous (IGIV)	Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC)	Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)	
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	
Number of subjects analysed	17	17	7	
Units: days				
median (full range (min-max))	0.00 (0.0 to 31.8)	0.00 (0.0 to 34.5)	0.00 (0.0 to 21.5)	

Statistical analyses

Statistical analysis title	Epoch 1: IGIV
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	4.01
Confidence interval	
level	95 %
sides	2-sided
lower limit	1.46
upper limit	8.54

Statistical analysis title	Epoch 2: IGSC
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)

Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	5.87
Confidence interval	
level	95 %
sides	2-sided
lower limit	2.32
upper limit	11.93

Statistical analysis title	Epoch 3: IGSC
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	3.07
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.37
upper limit	10.74

Secondary: Number of Participants Hospitalized due to Illness or Infection

End point title	Number of Participants Hospitalized due to Illness or Infection

End point description:

Number of participants with hospitalization are standardized to per year (365.25 days). A hospitalization is counted for a specific epoch only if that hospitalization started during that epoch. All-Treated Set included all enrolled participants who received at least 1 dose of study drug (IGIV or IGSC).

End point type	Secondary

End point timeframe:

From first dose of study drug up to end of study (up to approximately 1.5 years)

End point values	Epoch 1: Immune Globulin Intravenous (IGIV)	Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC)	Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)	
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	
Number of subjects analysed	17	17	7	
Units: participants	0	1	0	

Statistical analyses

Statistical analysis title	Epoch 1: IGIV
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	0
Confidence interval	
level	95 %
sides	2-sided
lower limit	0
upper limit	0

Statistical analysis title	Epoch 3: IGSC
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	0
Confidence interval	
level	95 %
sides	2-sided
lower limit	0
upper limit	0

Statistical analysis title	Epoch 2: IGSC
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)

Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	0.13
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.03
upper limit	0.35

Secondary: Length of Hospital Stay

End point title	Length of Hospital Stay

End point description:

Length of hospital stay per stay is standardized to per year (365.25 days). A hospitalization is counted for a specific epoch only if that hospitalization started during that epoch. All-Treated Set included all enrolled participants who received at least 1 dose of study drug (IGIV or IGSC).

End point type	Secondary

End point timeframe:

From first dose of study drug up to end of study (up to approximately 1.5 years)

End point values	Epoch 1: Immune Globulin Intravenous (IGIV)	Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC)	Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)	
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	
Number of subjects analysed	17	17	7	
Units: days				
median (full range (min-max))	0.00 (0.0 to 0.0)	0.00 (0.0 to 17.4)	0.00 (0.0 to 0.0)	

Statistical analyses

Statistical analysis title	Epoch 1: IGIV
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	0

Confidence interval	
level	95 %
sides	2-sided
lower limit	0
upper limit	0

Statistical analysis title	Epoch 3: IGSC
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	0
Confidence interval	
level	95 %
sides	2-sided
lower limit	0
upper limit	0

Statistical analysis title	Epoch 2: IGSC
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	1.04
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.25
upper limit	2.76

Secondary: Number of Acute Physician Visits due to Illness/Infection

End point title	Number of Acute Physician Visits due to Illness/Infection

End point description:

Number of acute physician visits is standardized to per year (365.25 days). All-Treated Set included all enrolled participants who received at least 1 dose of study drug (IGIV or IGSC).

End point type	Secondary

End point timeframe:

From first dose of study drug up to end of study (up to approximately 1.5 years)

End point values	Epoch 1: Immune Globulin Intravenous (IGIV)	Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC)	Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)	
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	
Number of subjects analysed	17	17	7	
Units: number of visits per year				
arithmetic mean (standard deviation)	1.18 (± 3.098)	3.30 (± 6.807)	0.61 (± 1.624)	

Statistical analyses

Statistical analysis title	Epoch 1: IGIV
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	1.18
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.38
upper limit	2.68

Statistical analysis title	Epoch 3: IGSC
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	0.61
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.07
upper limit	2.15

Statistical analysis title	Epoch 2: IGSC
Comparison groups	Epoch 1: Immune Globulin Intravenous (IGIV) v Epoch 2: Immune Globulin Subcutaneous 20% Solution (IGSC) v Epoch 3: Immune Globulin Subcutaneous 20% Solution (IGSC)
Number of subjects included in analysis	41
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Poisson Estimate
Point estimate	2.35
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.91
upper limit	4.82

Secondary: Health-related Quality of Life (HRQoL): Pediatric Quality of Life Inventory (PedsQL) Total Scale Score

End point title	Health-related Quality of Life (HRQoL): Pediatric Quality of Life Inventory (PedsQL) Total Scale Score

End point description:

Peds-QL=generic HR QoL instrument designed specifically for pediatrics has domains as:general health/activities,feelings/emotional,social functioning,school functioning.In this study,2-7 years (parent as observer),8-13 years (participant as observer) for Peds-QL health questionnaire was analyzed.Higher scores=better QOL for all domains.This modular instrument used 5-point scale:0(never) to 4(almost always).Items are reversed scored;linearly transformed to 0-100 scale as follows:0=100,1=75,2=50,3=25,4=0.4 dimensions(physical, emotional, social, & school functioning) are scored.PEDS-QL Total Scale Score has 0-100 scale,higher scores=better HRQoL. All-Treated Set included all enrolled participants of age group '2-7 years' and '8-13 years' who received at least 1 dose of study drug (IGIV or IGSC). 'n' indicates = Number analyzed are the number of participants with data available for analysis at the given timepoint.

End point type	Secondary

End point timeframe:

Baseline up to end of study (approximately 1.5 years)

End point values	Age 2-7 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-	Age 8-13years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	2	4		
Units: score on a scale				
arithmetic mean (standard deviation)				
IGIV: Epoch 1: Baseline (Week 1)(n=2,4)	88.04 (± 13.835)	87.23 (± 11.820)		
IGSC: Epoch 2: CFB (Week 1)(n=2,4)	9.78 (± 15.372)	-6.79 (± 9.977)		
IGSC: Epoch 3: CFB (Week 1)[n=1,1]	14.13 (± 999)	20.65 (± 999)		
IGSC: Epoch 3: CFB (EOS/ET)(n=2,4)	3.26 (± 7.686)	4.08 (± 16.374)		

Statistical analyses

No statistical analyses for this end point

Secondary: EuroQoL (Quality of Life)-5 Dimensions 3 Levels (EQ-5D-3L) Total Scale Score

End point title	EuroQoL (Quality of Life)-5 Dimensions 3 Levels (EQ-5D-3L) Total Scale Score

End point description:

EQ-5D-3L health questionnaire=participant answered questionnaire scoring 5 dimensions -mobility,self-care,usual activities, pain/discomfort and anxiety/depression. n this study, 2-11 years (parent as observer),12 years and older (participant as observer) for EQ-5D-3L health questionnaire was analyzed.Health state index score range from 0 (worst health state) to 1 (perfect health state) and 1 reflects the best outcome.EQ visual analogue scale range from 0 to 100, where higher scores indicate better health status.Data is reported as per age groups (2-11 years and >=12 years). All-Treated Set included all enrolled participants of age group '2-11 years' and '>=12 years' who received at least 1 dose of study drug (IGIV or IGSC). Number analyzed is the number of participants with data available for analyses at specific timepoints.

End point type	Secondary

End point timeframe:

Baseline up to end of the study (approximately 1.5 years)

End point values	Age 2-11years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-	Age ≥12 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	4	13		
Units: score on a scale				
arithmetic mean (standard deviation)				
IGIV: Epoch 1: Baseline (Week 1): HSIS(n=4,13)	0.8080 (± 0.04619)	0.8217 (± 0.04544)		
IGIV: Epoch 1: Baseline (Week 1): EQVAS(n=4,13)	87.5 (± 15.55)	77.1 (± 22.16)		
IGSC: Epoch 2: CFB (Week 1): HSIS (n=3,13)	-0.0270 (± 0.12275)	0.0016 (± 0.03318)		
IGSC: Epoch 2: CFB (Week 1): EQVAS (n=3,13)	-5.0 (± 5.00)	4.2 (± 17.15)		
IGSC: Epoch 3:CFB (Week 1): HSIS (n=1,6)	0.0000 (± 999)	0.0133 (± 0.03266)		
IGSC: Epoch 3:CFB (Week 1): EQVAS (n=1,6)	0.0 (± 999)	-1.3 (± 12.39)		
IGSC: Epoch 3:CFB (EOS/ET):HSIS(n=4,13)	0.0400 (± 0.04619)	0.0229 (± 0.04566)		
IGSC: Epoch 3:CFB (EOS/ET): EQVAS(n=4,13)	1.3 (± 6.29)	6.9 (± 13.21)		

Statistical analyses

No statistical analyses for this end point

Secondary: Health-related Quality of Life (HRQoL): Short Form-36 Health Survey (SF-36) Score

End point title	Health-related Quality of Life (HRQoL): Short Form-36 Health Survey (SF-36) Score

End point description:

SF-36=generic quality-of-life instrument that has been widely used to assess HRQL of participants.In this study, 14 years and older (participant as observer) for SF-36 health questionnaire was analyzed. Generic instruments are used in general populations to assess a wide range of domains applicable to a variety of health states, conditions, and diseases.SF-36=36 items aggregated into 8 multi-item scales (physical functioning, role - physical, bodily pain, general health, vitality, social functioning, role - emotional, and mental health), with scores ranging from 0 to 100.Higher scores=better HRQL. As pre-specified in protocol data is reported for participants with age group of 14 years or older.All-Treated Set included all enrolled participants of age '14 years and above' who received at least 1 dose of study drug (IGIV or IGSC).'n'=Number analysed are participants with data available for analysis.Physical Component Summary Score (PCSS), Mental Component Summary Score (MCSS).

End point type	Secondary

End point timeframe:

Baseline up to end of the study (approximately 1.5 years)

End point values	Age ≥14 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-			
Subject group type	Subject analysis set			
Number of subjects analysed	11			
Units: score on a scale				
arithmetic mean (standard deviation)				
IGIV: Epoch 1: Baseline (Week 1): PCSS(n=11)	50.21 (± 7.282)			
IGIV: Epoch 1: Baseline (Week 1): MCSS(n=11)	50.59 (± 8.114)			
IGSC: Epoch 2: CFB (Week 1): PCSS(n=11)	0.10 (± 7.142)			
IGSC: Epoch 2: CFB (Week 1): MCSS(n=11)	-0.30 (± 2.294)			
IGSC: Epoch 3: CFB (Week 1): PCSS(n=5)	1.81 (± 8.189)			
IGSC: Epoch 3: CFB (Week 1): MCSS (n=5)	3.60 (± 5.352)			
IGSC: Epoch 3:CFB (EOS/ET):PCSS(n=10)	0.23 (± 4.649)			
IGSC: Epoch 3:CFB (EOS/ET):MCSS(n=10)	4.32 (± 8.185)			

Statistical analyses

No statistical analyses for this end point

Secondary: Health Related Quality of Life: Treatment Satisfaction Questionnaire for Medication-9 (TSQM-9) Score

End point title	Health Related Quality of Life: Treatment Satisfaction Questionnaire for Medication-9 (TSQM-9) Score

End point description:

TSQM=is a global satisfaction scale used to assess the overall level of participant's satisfaction or dissatisfaction with their medications. In this study, 2-12 years (parent as observer), 13 years and older (participant as observer) for TSQM health questionnaire will be analyzed. TSQM-9 is a 9-item, validated, self-administered instrument used to assess participant's satisfaction with medication. The three domains assessed are effectiveness, convenience, and global satisfaction. The score of each of the 3 domains is based on an algorithm to create a score of 0 to 100. Higher score indicated greater satisfaction in that domain. As pre-specified in protocol, data is reported as per age group (2-12 years and >=13 years). All-Treated Set included all enrolled participants of age group ' 2-12 years' and '>=3 years' who received at least 1 dose of study drug (IGIV or IGSC). 'n' indicates number analyzed is the number of participants with data available for analyses at specific timepoints.

End point type	Secondary

End point timeframe:

Baseline up to end of the study (approximately 1.5 years)

End point values	Age 2-12years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-	Age ≥13 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	6	11		
Units: score on a scale				
arithmetic mean (standard deviation)				
IGIV:Epoch1:Baseline(Week1):Effectiveness(n=6,11)	69.44 (± 14.380)	75.25 (± 18.817)		
IGIV:Epoch 1:Baseline(Week 1):Convenience(n=6,11)	62.96 (± 13.907)	65.66 (± 23.016)		
IGIV: Epoch 1: Baseline (Week 1): GS(n=6,11)	75.00 (± 17.928)	74.68 (± 18.173)		
IGSC: Epoch 2: CFB (Week 1): Effectiveness(n=5,11)	2.22 (± 9.296)	-1.52 (± 10.568)		
IGSC: Epoch 2: CFB (Week 1): Convenience (n=5,11)	3.33 (± 16.942)	5.05 (± 13.484)		
IGSC: Epoch 2: CFB (Week 1): GS (n=5,11)	-2.86 (± 10.833)	1.30 (± 10.507)		
IGSC: Epoch 3: CFB (Week 1): Effectiveness (n=2,5)	2.78 (± 3.928)	-10.00 (± 52.673)		
IGSC: Epoch 3: CFB (Week 1): Convenience (n=2,5)	13.89 (± 19.642)	15.56 (± 12.669)		

IGSC: Epoch 3: CFB (Week 1): GS (n=2,5)	-7.14 (± 10.02)	4.29 (± 29.709)		
IGSC:Epoch 3:CFB (EOS/ET):Effectiveness(n=6,11)	9.26 (± 10.924)	6.57 (± 18.892)		
IGSC: Epoch 3:CFB (EOS/ET): Convenience (n=6,11)	5.56 (± 22.222)	9.09 (± 23.210)		
IGSC: Epoch 3: CFB (EOS/ET): GS(n=6,11)	7.14 (± 23.905)	3.90 (± 23.768)		

Statistical analyses

No statistical analyses for this end point

Secondary: Health Related Quality of Life: Treatment Satisfaction Questionnaire for Life Quality Index (LQI) Score

End point title	Health Related Quality of Life: Treatment Satisfaction Questionnaire for Life Quality Index (LQI) Score

End point description:

LQI=self-administered questionnaire developed specifically for participants/legal guardians involved in IGIV treatments.2-13 years (parent as observer),14 years and older (participant as observer) for LQI health questionnaire was analyzed.LQI=15-items, divided into 4 domains: treatment interferences(TI)[6 items],therapy-related problems(TRP)[4 items],therapy setting(TS)[3 items];treatment costs(TC)[2 items].Items are rated on a 7-point Likert-type scale ranging from 1:"Extremely bad" to 7:"Extremely good".Total scores=0 to 100,higher scores=highest possible satisfaction with factors such as independence,therapy convenience,social/school/work activities;health and travel costs.As pre-specified in protocol, data is reported as per age (2-13 years and >=14 years).All-Treated Set included all enrolled participants of age group '2-13 years' and '>=14 years' who received at least 1 dose of study drug (IGIV or IGSC).'n'=Number analysed are participants with data available for analysis.

End point type	Secondary

End point timeframe:

Baseline up to end of the study (approximately 1.5 years)

End point values	Age 2-13years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-	Age ≥14 years:Epoch 1+2+3[IGIV200-600+IGSC50-100+100-		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	6	11		
Units: score on a scale				
arithmetic mean (standard deviation)				
IGIV:Epoch 1:Baseline(Week 1):Factor 1 TI(n=6,11)	81.48 (± 14.873)	77.02 (± 17.963)		
IGIV:Epoch1:Baseline(Week1):Factor 1 TRP(n=6,11)	81.94 (± 15.516)	79.17 (± 15.811)		
IGIV:Epoch 1:Baseline(Week 1):Factor 1 TS(n=6,11)	77.78 (± 20.488)	74.75 (± 23.748)		
IGIV:Epoch 1:Baseline(Week 1):Factor 1 TC(n=6,11)	65.28 (± 24.391)	53.79 (± 21.847)		
IGSC: Epoch 2: CFB (Week 1): Factor 1 TI(n=6,11)	-8.33 (± 21.445)	-2.53 (± 14.351)		
IGSC: Epoch 2: CFB (Week 1): Factor 1 TRP(n=6,11)	2.78 (± 9.742)	-1.52 (± 15.841)		

IGSC: Epoch 2: CFB (Week 1): Factor 1 TS(n=6,11)	-6.48 (± 30.106)	0.51 (± 11.773)		
IGSC: Epoch 2: CFB (Week 1): Factor 1 TC(n=6,11)	-9.72 (± 22.618)	0.76 (± 31.282)		
IGSC: Epoch 3: CFB (Week 1): Factor 1 TI (n=5,5)	4.17 (± 1.964)	3.33 (± 4.120)		
IGSC: Epoch 3: CFB (Week 1): Factor 1 TRP (n=5,5)	-14.58 (± 14.731)	1.67 (± 14.613)		
IGSC: Epoch 3: CFB (Week 1): Factor 1 TS (n=5,5)	19.44 (± 3.928)	12.22 (± 16.387)		
IGSC: Epoch 3: CFB (Week 1): Factor 1 TC (n=5,5)	0.00 (± 0.000)	18.33 (± 40.139)		
IGSC:Epoch3:CFB(Week13[EOS/ET]):Factor1 TI(n=6,11)	-2.78 (± 13.494)	-0.25 (± 13.046)		
IGSC:Epoch3:CFB(Week13[EOS/ET]):Factor1TRP(n=6,11)	-1.39 (± 9.001)	1.14 (± 9.334)		
IGSC:Epoch3:CFB(Week 13[EOS/ET]):Factor1TS(n=6,11)	2.78 (± 16.005)	4.55 (± 16.067)		
IGSC:Epoch3:CFB(Week 13[EOS/ET]):Factor1TC(n=6,11)	4.17 (± 16.672)	14.39 (± 26.898)		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

From first dose of study drug up to end of treatment (up to Week 57)

Adverse event reporting additional description:

At each visit the investigator had to document any occurrence of adverse events and abnormal laboratory findings. Any event spontaneously reported by the participant or observed by the investigator was recorded, irrespective of the relation to study treatment.

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	24.0

Reporting groups

Reporting group title	Epoch 1: IGIV 200-600 mg/kg

Reporting group description:

Participants received 200 to 600 mg/kg of Immunoglobulin Intravenous (IGIV) infusion for every 3 or 4 weeks for up to 13 weeks.

Reporting group title	Epoch 3: IGSC (20%) 100-400 mg/kg

Reporting group description:

Participants who entered to Epoch 3 from Epoch 2 received 100-400 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion biweekly up to approximately 12 weeks after Epoch 2.

Reporting group title	Epoch 2: IGSC (20%) 50-200 mg/kg

Reporting group description:

Participants who entered to Epoch 2 from Epoch 1 received 50-200 mg/kg of Immune Globulin Subcutaneous (Human) 20% infusion once a week up to approximately 24 weeks after Epoch 1.

Serious adverse events	Epoch 1: IGIV 200-600 mg/kg	Epoch 3: IGSC (20%) 100-400 mg/kg	Epoch 2: IGSC (20%) 50-200 mg/kg
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	0 / 17 (0.00%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0

Frequency threshold for reporting non-serious adverse events: 5 %

Non-serious adverse events	Epoch 1: IGIV 200-600 mg/kg	Epoch 3: IGSC (20%) 100-400 mg/kg	Epoch 2: IGSC (20%) 50-200 mg/kg
Total subjects affected by non-serious adverse events			
subjects affected / exposed	11 / 17 (64.71%)	3 / 7 (42.86%)	16 / 17 (94.12%)
Investigations			

Blood creatine phosphokinase increased			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Occult blood positive			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Colon adenoma			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Seborrhoeic keratosis			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Injury, poisoning and procedural complications			
Vaccination complication			
subjects affected / exposed	0 / 17 (0.00%)	1 / 7 (14.29%)	1 / 17 (5.88%)
occurrences (all)	0	1	2
Ligament sprain			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Wound			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Traumatic arthropathy			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Nervous system disorders			
Headache			
subjects affected / exposed	3 / 17 (17.65%)	1 / 7 (14.29%)	3 / 17 (17.65%)
occurrences (all)	7	1	8
Orthostatic intolerance			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Sciatica			

subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
General disorders and administration site conditions			
Injection site swelling			
subjects affected / exposed	0 / 17 (0.00%)	1 / 7 (14.29%)	3 / 17 (17.65%)
occurrences (all)	0	1	7
Injection site erythema			
subjects affected / exposed	0 / 17 (0.00%)	1 / 7 (14.29%)	3 / 17 (17.65%)
occurrences (all)	0	1	4
Injection site pain			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	2 / 17 (11.76%)
occurrences (all)	0	0	2
Pyrexia			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	1	0	2
Administration site discolouration			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Vaccination site pain			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	2 / 17 (11.76%)
occurrences (all)	0	0	2
Administration site pain			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	3
Administration site swelling			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Chills			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Infusion site bruising			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Infusion site erythema			

subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	4
Infusion site pain			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Infusion site pruritus			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	4
Infusion site swelling			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	7
Injection site bruising			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Vaccination site joint erythema			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Puncture site pain			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Malaise			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	2
Injection site reaction			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Eye disorders			
Myopia			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Gastrointestinal disorders			
Stomatitis			
subjects affected / exposed	3 / 17 (17.65%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	4	0	1
Dental caries			

subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	1	0	1
Diarrhoea			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	2	0	1
Nausea			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	1	0	1
Constipation			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Gastrooesophageal reflux disease			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Toothache			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Haemorrhoids			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Skin and subcutaneous tissue disorders			
Acne			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Eczema asteatotic			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Dermatitis atopic			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Miliaria			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Rash pruritic			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1

Seborrhoeic dermatitis			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Urticaria			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Psychiatric disorders			
Insomnia			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Musculoskeletal and connective tissue disorders			
Pain in extremity			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	2 / 17 (11.76%)
occurrences (all)	0	0	2
Arthritis			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Pain in jaw			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Back pain			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	2	0	0
Infections and infestations			
Conjunctivitis			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	2 / 17 (11.76%)
occurrences (all)	1	0	2
Nasopharyngitis			
subjects affected / exposed	2 / 17 (11.76%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	2	0	2
Gastroenteritis			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	2 / 17 (11.76%)
occurrences (all)	0	0	2
Sinusitis			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	2 / 17 (11.76%)
occurrences (all)	0	0	3

Upper respiratory tract infection			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	1	0	1
Bronchitis			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Cystitis			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Hordeolum			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Impetigo			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Laryngitis			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Oral herpes			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Otitis externa			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Parotitis			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1
Pharyngitis			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Periodontitis			
subjects affected / exposed	1 / 17 (5.88%)	0 / 7 (0.00%)	0 / 17 (0.00%)
occurrences (all)	1	0	0
Pneumonia			
subjects affected / exposed	0 / 17 (0.00%)	0 / 7 (0.00%)	1 / 17 (5.88%)
occurrences (all)	0	0	1

Pulpitis dental subjects affected / exposed occurrences (all)	0 / 17 (0.00%) 0	0 / 7 (0.00%) 0	1 / 17 (5.88%) 1

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported